

## June 2013 Newsletter

### CIRM June Newsletter

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### CIRM Awards Sangamo Over \$6 Million Toward a Clinical Trial for a Stem Cell-Based Blood Disorder Therapy



At its May 23rd meeting, the CIRM governing board approved \$6.37 million in funding for Sangamo BioSciences, as part of the Strategic Partnership II awards. Sangamo will match that amount to help develop a potential therapy for beta-thalassemia, a disorder that limits the ability of blood cells to carry oxygen around the body leading to anemia and other serious complications. During the four-year award the company is expected to finish a phase 1 clinical trial for their proposed therapy.

- [Read a summary of the project](#)
- [Learn more about CIRM funding for thalassemia](#)
- [Read our press release](#)
- [Read Sangamo's Press Release](#)

### Governing Board Approves \$36 Million to Help Recruit World-Class Stem Cell Scientists to California

Also at its May meeting, the CIRM governing board awarded \$36 million in funds to attract six world-class scientists to the state. These Research Leadership Awards were created by CIRM to help California universities and research institutions recruit the very best stem cell scientists in the world. These awards have so far helped California institutions recruit four other senior scientists to the state.

- [Read our press release for a list of the recruited scientists](#)
- [See all Research Leadership grantees](#)

### Oregon Research Team Derives Embryonic Stem Cells via Nuclear Transfer

On May 15th, a team at the Oregon Health & Science University published a report in the journal *Cell* that they had successfully derived six embryonic stem cell lines from embryos created by somatic cell nuclear transfer (SCNT), also called cloning. CIRM President Alan Trounson discussed the milestone achievement in his monthly stem cell research report. Here is an excerpt:

"Teams around the world have tried to create cell lines like these ever since Jamie Thomson first isolated human embryonic stem cells from unused IVF embryos in 1998. Starting with a donated human egg, you remove its chromosomes, and then replace this genetic material with chromosomes from an adult cell, get the egg to start to mature and on day five or six harvest stem cells. While this had been accomplished in many animal species, repeating the technique in human had been illusive.

After the paper came out some concerns were raised about errors involving duplication of figures. The resulting cell lines need to be independently analyzed by geneticists who should be able to detect DNA from the donor egg and the adult cell. However, I do think the result will stand and open the door to some critical comparative analysis. We can now create reprogrammed iPS cells

and embryonic cells from the same donor giving us the opportunity to compare the genetic stability of the two types of cells. New reprogramming factors found in the eggs could help improve the efficiency of creating iPS cells."

- Read our blogs [here](#) and [here](#) for more discussion about the paper

#### **New Webinar: Moving Stem Cell-Based Therapies to the Clinic**

## **A Phase 1 trial of spinal cord transplantation of neural progenitor cells in ALS (Neuralstem)**



***Jonathan D. Glass  
Emory University  
Atlanta, Georgia USA***



On April 15th, CIRM hosted a webinar presentation entitled, "Clinical Trials: Moving Stem Cell based Therapies to the Clinic". The webinar is now available for viewing on CIRM's website. The focus of the webinar was on the lessons learned from Investigational New Drug (IND)/Phase I trials including overcoming the challenges, and addressing the regulatory issues, as stem cell based therapies move towards the clinic. Ellen Feigal, M.D., CIRM's Senior VP of Research and Development moderated the webinar which included a presentation by the FDA as well as discussions of Phase 1 stem cell-based trials for heart failure and ALS.

- Read agenda

#### **CIRM Grantee Marius Wernig Named 2013 Outstanding Young Investigator by ISSCR**



The International Society for Stem Cell Research announced that CIRM grantee Marius Wernig of Stanford University is the recipient of its fifth annual Outstanding Young Investigator Award in 2013. This award is particularly in recognition of his research that shows a given cell type can be directly reprogrammed into an unrelated cell type. Wernig is part of a CIRM disease team working on a therapy for a deadly childhood skin disease called epidermolysis bullosa, and has a Tools and Technologies award to study neurological disorders by converting skin cells directly into nerves.

- [Read our blog about Wernig's award](#)

### **CIRM President Alan Trounson's Stem Cell Research Picks**

Each month CIRM President Alan Trounson reports on advances in stem cell science by highlighting recently published papers from CIRM grantees and other leading research teams around the world. The top story of his May picks describes the successful derivation of embryonic stem cell lines from embryos created by somatic cell nuclear transfer (SCNT). Also included are published results on stem cell-based research related to Down's Syndrome and epilepsy as well as a report on creating a functioning human thymus from stem cells.

- [Read about all the president's picks in his report](#)

### **1300 and Counting: The Latest Publications on CIRM-funded Research**

With over 1300 published discoveries, CIRM grantees are making steady progress on understanding the molecular basis of stem cell biology and moving stem cell-based therapies toward clinical trials. Here is a partial list of science journals that published CIRM-funded research in May along with press releases from the grantees' home institutions and entries from the CIRM Stem Cell Research Blog:

**May 2nd - *Cell Stem Cell***  
**May 5th - *Nature Neuroscience***

- UCSF Press Release: Human Brain Cells Developed in Lab, Grow in Mice
- CIRM Blog: Transplanted neurons treat mice with epilepsy

**May 5th - *Nature Cell Biology***

- Stanford Press Release: Blocking protein expression delays onset of multiple sclerosis in mice, study says
- CIRM Blog: Getting rid of a protein that promotes longer lifespan slows MS in mice

**May 8th - *Nature Communications***

- UCLA Press Release: UCLA stem cell researchers move toward treatment for rare genetic nerve disease
- CIRM Blog: Stem cells model rare neurological disease, suggest possible therapy

**May 16th - *Advanced Healthcare Materials***

- City of Hope Press Release: Tiny gold rods could be used to attack hard-to-reach cancers
- CIRM Blog: Stem cells carrying gold nanoparticles could treat tumors, spare healthy tissue

### May 28th - *Stem Cell Research & Therapy*

- UCSD Press Release: Stem Cell Injections Improve Spinal Injuries in Rats
- CIRM Blog: CIRM grantees at UCSD use stem cell injections to improve spinal cord injuries in rats

### Upcoming CIRM Events



**June 12th-15th:** The International Society for Stem Cell Research (ISSCR) holds its 11th annual meeting in Boston. Visit the meeting's home page for more details.

**June 20th, 10-11am Pacific Time:** CIRM **hosts a free webinar**, "Reimbursement Strategies: Planning Your Payment Strategy in Early Product Development". Visit the agenda page for more details including free registration information.

**July 25th:** The CIRM governing board will hold its 4th meeting of 2013 at the Hilton Hotel in Burlingame. The agenda and details will be posted to the **meetings page** 10 days before the event.

### CIRM Funding Opportunities

**RFA 13-02: CIRM Basic Biology Awards V** - Full Applications due 5:00 pm (PDT), June 27, 2013. Only pre-applicants invited to submit a full application are eligible.

**PA 12-08: CIRM Patent Assistance Fund Awards** - Application opportunities on a monthly basis until June 30, 2014

**Eligibility:** Only Technology Transfer Officials at current CIRM Grantee institutions are eligible to apply for funds under this program.

Get more info about CIRM RFAs

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